Praxis Precision Medicines Receives PRIME Designation from the EMA for elsunersen (PRAX-222) for Treatment of SCN2A Gain of Function Developmental Epilepsies

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BOSTON, Nov. 16, 2023 (GLOBE NEWSWIRE) -- Praxis Precision Medicines, Inc. (NASDAQ: PRAX), a clinical-stage biopharmaceutical company translating genetic insights into the development of therapies for central nervous system (CNS) disorders characterized by neuronal excitation-inhibition imbalance, today announced that the European Medical Agency (EMA) has awarded its Priority Medicines (PRIME) designation for elsunersen (PRAX-222) for the treatment of SCN2A Gain of Function (GoF) developmental and epileptic encephalopathy (DEE). The EMA's PRIME designation provides enhanced development support for priority medicines that target an unmet need and was granted based on the Part 1 data from the EMBRAVE study that showed a reduction in seizures and improvement in seizure free days, as well as preclinical data.

"Elsunersen has the potential to significantly impact the lives of patients with SCN2A-DEE and their families," said Marcio Souza, president and chief executive officer of Praxis. "We welcome the recognition by the EMA of not only the unmet need in the condition, but the breakthrough potential of elsunersen and look forward to working closely with patients and regulators globally to advance the program."

The PRIME designation provides early and proactive support to developers of promising medicines that may offer a major therapeutic advantage over existing treatments or a benefit to patients without treatment options. These medicines are considered priority medicines by the EMA, whose aim is to optimize development plans and accelerate evaluations so medicines that address significant unmet medical needs can reach patients faster.

About the EMBRAVE Study
Part 1 of the EMBRAVE study is an open-label cohort involving pediatric patients aged 2 to18 years. These patients, diagnosed with early-onset SCN2A developmental and epileptic encephalopathy (SCN2A-DEE), are administered elsunersen over a period of up to 13 weeks. The primary objective is to determine the safety and tolerability of intrathecal delivery of elsunersen. To learn more about the EMBRAVE study, please visit https://www.embravestudy.com/.

About elsunersen (PRAX-222)
Elsunersen is an antisense oligonucleotide (ASO) designed to selectively decrease SCN2A gene expression, directly targeting the underlying cause of early-seizure-onset SCN2A-DEE to treat seizures and other symptoms in patients with gain-of-function SCN2A mutations. In vitro studies of elsunersen have demonstrated reduction in both SCN2A gene expression and protein levels. In vivo, elsunersen has demonstrated significant, dose-dependent reduction in seizures, improvement in behavioral and locomotor activity and increased survival in SCN2A mouse models, with potential to be the first disease-modifying treatment for SCN2A-DEE. Elsunersen has received Orphan Drug Designation (ODD) and Rare Pediatric Disease Designation (RPD) from the FDA, and ODD from the European Medicines Agency (EMA) for the treatment of SCN2A-DEE. The elsunersen program is ongoing under a collaboration with Ionis Pharmaceutics, Inc. (NASDAQ: IONS), and RogCon, Inc.

About SCN2A-DEE
SCN2A developmental and epileptic encephalopathy (SCN2A-DEE) is a debilitating monogenic epilepsy disorder caused by a variant in the SCN2A gene, associated with early mortality. The SCN2A gene is critical in the formation of sodium channel proteins in the brain, which control the flow of sodium ions into neurons. This movement of sodium ions is a major component of generating electrical signals called action potentials, the way in which the cells communicate. SCN2A-DEE is characterized by a broad spectrum of phenotypes. Early-onset SCN2A-DEE presents before three months and can lead to profound impact on patients, including drug-resistant seizures, significant cognitive impairment, movement disorders such as dystonia or ataxia and problems in other body systems such as gastrointestinal or ocular. Currently there are no approved treatments for SCN2A-DEE, and the standard-of-care typically involves a regimen of many concurrent anti-seizure medications as well as medications to manage co-morbidities. Despite these interventions, more than 70% of early-onset SCN2A-DEE patients live with uncontrolled seizures, and approximately 75% live with severe intellectual disability with patients rarely surviving beyond their teenage years.

About Praxis
Praxis Precision Medicines is a clinical-stage biopharmaceutical company translating insights from genetic epilepsies into the development of therapies for CNS disorders characterized by neuronal excitation-inhibition imbalance. Praxis is applying genetic insights to the discovery and development of therapies for rare and more prevalent neurological disorders through our proprietary small molecule platform, Cerebrum™, and antisense oligonucleotide (ASO) platform, Solidus™, using our understanding of shared biological targets and circuits in the brain. Praxis has established a diversified, multimodal CNS portfolio including multiple programs across movement disorders and epilepsy, with four clinical-stage product candidates. For more information, please visit www.praxismedicines.com and follow us on Facebook, LinkedIn and Twitter/X.

Investor Contact: Praxis Precision Medicines investors@praxismedicines.com 857-702-9452 Media Contact: Ian Stone Canale Communications Ian.stone@canalecomm.com 619-849-5388